DIA

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HYATT REGENCY BETHESDA • ONE BETHESDA METRO CENTER • BETHESDA, MD, USA

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APPLICATION AND VALIDATION OF GENOMIC BIOMARKERS FOR USE IN DRUG DEVELOPMENT AND REGULATORY SUBMISSIONS

Co-sponsors

DIA
Drug Information Association

FDA
US Food and Drug Administration

PhRMA
Pharmaceutical Research and Manufacturers of America

PWG
Pharmacogenetics Working Group

BIO
Biotechnology Industry Organization

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Welcome !

- Special Thanks to:
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  - Allan Rudman, PhD
  - Federico Goodsaid, PhD
  - All members of the Organizing Committee
  - Leadership at FDA, in particular:
    - Janet Woodcock, MD
    - Larry Lesko, PhD

FDA-DIA-Industry Workshops: A Tradition in the Making

Why Are We Here ?

"The future belongs to those who see possibilities before they become obvious."

– John Sculley

FDA's Mission Statement

1. The FDA is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, our nation's food supply, cosmetics, and products that emit radiation.
2. The FDA is also responsible for advancing the public health by helping to speed innovations that make medicines and foods more effective, safer, and more affordable; and helping the public get the accurate, science-based information they need to use medicines and foods to improve their health.

FDA's Mission & Biomarkers

1. Safety, Efficacy:
   - The use of biomarkers will change medical practice from a population-based approach to an individualized approach: molecular and patho-physiological characteristics of an individual patient will be measured and (drug) therapy will be tailored to individual needs.
2. Innovations:
   - The use of biomarkers will enhance the way drugs are being developed: new, innovative approaches will utilize the knowledge of molecular mechanisms to develop better drugs for a more targeted market.

Lifecycle of a Biomarker

1. Identification
2. Qualification (analytical, clinical)
3. Integration
   - Context of prediction
   - Added value at lower cost than alternatives
4. Use in Drug Development
5. More use in Clinical Practice

Genomic Biomarkers

- Guidance on “Pharmacogenomic Data Submissions” introduces a classification system for distinguishing between the extent of qualification of genomic biomarkers:
  - Known valid
  - Probable valid
  - Exploratory
- But how valid is *my* biomarker ?

Focus of Workshop

1. Identification
2. Qualification (analytical, clinical)
3. Integration
   - Context of prediction
   - Added value at lower cost than alternatives
4. Use in Drug Development
5. More use in Clinical Practice

Program: Day One

- R & D
  1. Two keynotes introducing the use of biomarkers in research and development
  2. Sessions on safety and efficacy biomarkers
  3. Break-out sessions
     - Standards
     - Validation
  4. Keynote to put it in perspective and look ahead

Program: Day Two

- Regulation
  1. Keynote introducing the use of biomarkers in regulation
  2. Session on establishing a regulatory framework for biomarker development
  3. Case studies (collaboration, VGDS)
  4. Break-out sessions
     - Genomic biomarkers and regulatory decision making
     - Databases for safety and efficacy biomarkers

Desired Outcome

- Definition of process and standards to qualify a genomic biomarker for a stated purpose ~ “vetting” of a qualification protocol
- Efficient and cost-effective strategies to incorporate genomic biomarkers in drug development programs
- Goal: To prepare a draft concept paper on qualification of genomic biomarkers for further public discussion

Let's Get Started !

"To grasp and hold a vision, that is the very essence of successful leadership — not only on the movie set where *I* learned it, but everywhere."

– Ronald Reagan